



Make today a breakthrough.

March 12, 2019

Senator Mary Daugherty Adams
Representative Jonathan Steinberg
Co-Chairs
Connecticut Public Health Committee
Legislative Office Building, Room 3000
Hartford, CT 06106

Dear Senator Adams and Representative Steinberg:

On behalf of the largest nonprofit organization dedicated to finding a cure for spinal muscular atrophy (SMA), we ask you to approve HB 7282 and include SMA on Connecticut's newborn screening panel.

SMA is the most common genetic cause of death in infants in the United States, affecting approximately 1 in 11,000 newborns. The condition is caused by a mutation in the survival motor neuron gene 1 (*SMN1*). In a healthy person, this gene produces a protein that is critical to the function of the nerves that control our muscles. Without it, those nerve cells cannot properly function and eventually die, leading to debilitating and often fatal muscle weakness. Without treatment, SMA type 1, the most common and severe form of the condition, results in death or permanent ventilation before two years of age. While other forms of SMA are not as severe, they all cause profound loss of muscle function and ability.

Newborn screening is particularly important to treating SMA. The FDA approved treatment, SPINRAZA, must be administered as soon as possible to be most effective. In fact, in clinical trials of SPINRAZA, pre-symptomatic infants with the genetic markers causing SMA are reaching age-appropriate developmental milestones. To date, none of the infants treated before showing symptoms have died. We expect the FDA to approve another treatment this spring, but it too must be administered quickly to achieve the best health outcomes possible.

Given the importance of newborn screening in effectively treating SMA, Health and Human Services Secretary Alex Azar added SMA to the Recommended Uniform Screening Panel in July of 2018. Twenty other states have approved adding SMA to their newborn testing program, and five states have already begun testing.



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For further information, please contact Jaimie Vickery, Vice President of Policy and Advocacy for Cure SMA at 202-841-4001 or at jaimie.vickery@curesma.org. We look forward to working with you to ensure the best possible health outcomes for Connecticut's newborns and their families.

Sincerely,

Handwritten signature of Kenneth Hobby in black ink.

Kenneth Hobby
President

Handwritten signature of Jill Jarecki in black ink.

Jill Jarecki, PhD
Chief Scientific Officer

Handwritten signature of Mary Schroth in black ink.

Mary Schroth, M.D.
Chief Medical Officer

Handwritten signature of Jaimie Vickery in black ink.

Jaimie Vickery
Vice President, Advocacy
and Policy